

## Food and Drug Administration, HHS

## § 316.26

marketing approval was for a rare disease or condition.

[78 FR 35133, June 12, 2013]

### § 316.24 Deficiency letters and granting orphan-drug designation.

(a) FDA will send a deficiency letter to the sponsor if the request for orphan-drug designation lacks information required under §§ 316.20 and 316.21, or contains inaccurate or incomplete information. FDA may consider a designation request voluntarily withdrawn if the sponsor fails to respond to the deficiency letter within 1 year of issuance of the deficiency letter, unless within that same timeframe the sponsor requests in writing an extension of time to respond. This request must include the reason(s) for the requested extension and the length of time of the requested extension. FDA will grant all reasonable requests for an extension. In the event FDA denies a request for an extension of time, FDA may consider the designation request voluntarily withdrawn. In the event FDA considers a designation request voluntarily withdrawn, FDA will so notify the sponsor in writing.

(b) FDA will grant the request for orphan-drug designation if none of the reasons described in § 316.25 for requiring or permitting refusal to grant such a request applies.

(c) When a request for orphan-drug designation is granted, FDA will notify the sponsor in writing and will publicize the orphan-drug designation in accordance with § 316.28.

(d) A sponsor may voluntarily withdraw an orphan-drug designation request or an orphan-drug designation at any time after the request is submitted or granted, respectively, by submitting a written request for withdrawal to FDA. FDA will acknowledge such withdrawal in a letter to the sponsor. Any benefits attendant to designation (such as orphan-exclusive approval) will cease once designation is voluntarily withdrawn, from the date of FDA's acknowledgement letter. If a sponsor voluntarily withdraws designation, FDA will publicize such withdrawal in accordance with § 316.28.

[57 FR 62085, Dec. 29, 1992, as amended at 78 FR 35133, June 12, 2013]

### § 316.25 Refusal to grant orphan-drug designation.

(a) FDA will refuse to grant a request for orphan-drug designation if any of the following reasons apply:

(1) The drug is not intended for a rare disease or condition because:

(i) There is insufficient evidence to support the estimate that the drug is intended for treatment of a disease or condition in fewer than 200,000 people in the United States, or that the drug is intended for use in prevention or in diagnosis in fewer than 200,000 people annually in the United States; or

(ii) Where the drug is intended for prevention, diagnosis, or treatment of a disease or condition affecting 200,000 or more people in the United States, the sponsor has failed to demonstrate that there is no reasonable expectation that development and production costs will be recovered from sales of the drug for such disease or condition in the United States. A sponsor's failure to comply with § 316.21 shall constitute a failure to make the demonstration required in this paragraph.

(2) There is insufficient information about the drug, or the disease or condition for which it is intended, to establish a medically plausible basis for expecting the drug to be effective in the prevention, diagnosis, or treatment of that disease or condition.

(3) The drug is otherwise the same drug as an already approved drug for the same rare disease or condition and the sponsor has not submitted a medically plausible hypothesis for the possible clinical superiority of the subsequent drug.

(b) FDA may refuse to grant a request for orphan-drug designation if the request for designation contains an untrue statement of material fact or omits material information or if the request is otherwise ineligible under this part.

[57 FR 62085, Dec. 29, 1992, as amended at 78 FR 35133, June 12, 2013]

### § 316.26 Amendment to orphan-drug designation.

(a) At any time prior to approval of a marketing application for a designated orphan drug, the sponsor holding designation may apply for an amendment to the designated use if the proposed